Sickle Cell Disease Fact Sheet

CIRM funds many projects seeking to better understand sickle cell disease and to translate those discoveries into new therapies.

Description

More than 80,000 Americans have sickle cell disease and despite decades of research the average life expectancy has dropped from 42 in 1995 to 39 today. It is a disease that largely targets the African-American community and to a lesser degree the Hispanic community.

Sickle cell disease is a genetic disorder that causes red blood cells to assume a sickle shape under stress, clogging blood vessels and producing episodes of excruciating pain, called crises, and leading to progressive organ damage. By twenty years of age about 15 percent of people with sickle cell disease have had major strokes and by 40 almost half of the patients have significant mental dysfunction.

The most common recommendation for people with sickle cell disease is to stay hydrated. The more water a person drinks, the less likely it is that their abnormal blood cells will clog their blood vessels. Another effective treatment is a medication called hydroxyurea, which reduces crises by 50 percent and death by 40 percent, but most adults are not treated. The populations most effected by sickle cell disease also suffer from significant health care disparities, which lower the quality of care they receive for their disease.

Bone marrow transplants are used to treat children with the most severe cases of the disease. In fact one of CIRM's board members, Bert Lubin, MD, the President and CEO of Children's Hospital and Research Center Oakland, has been a leader in developing this therapy for kids with sickle cell disease (his bio is here). The replacement bone marrow cells generate an entirely new blood system for the patient. However, bone marrow transplants are extremely risky and require a matched sibling donor and even under the best conditions there is always the risk of rejection.

Research funded by California's stem cell agency focuses on making bone marrow transplants safer and more effective for treating people with sickle cell disease. In one project, the researchers intend to remove bone marrow from the patient and fix the genetic defect in the blood-forming stem cells. Then those cells can be reintroduced into the patient to create a new, healthy blood system. Because the cells come from the patient this technique avoids the issue of rejection. Other researchers are developing ways of making bone marrow transplants safer.

Clinical Stage Programs

University of California, Los Angeles

This team of researchers plans to remove bone marrow cells from people with sickle cell disease and fix the genetic mutation that causes the disease. The team will then reintroduce the new cells into the patient. Those cells will then generate new, healthy blood cells.

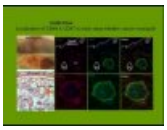
- Read more about this project

CIRM Grants Targeting Sickle Cell Disease

Researcher name	Institution	Grant Title	Grant Type	Approved funds
Hanna Mikkola	University of California, Los Angeles	Mechanisms of Hematopoietic stem cell Specification and Self-Renewal	New Faculty I	$2,286,900

Donald Kohn	University of California, Los Angeles	Clinical Trial of Stem Cell Gene Therapy for Sickle Cell Disease	Disease Team Therapy Development III	$13,145,465	
Donald Kohn	Children's Hospital of Los Angeles	STEM CELL GENE THERAPY FOR SICKLE CELL DISEASE	Disease Team Planning	$12,131	
Mark Walters	Children's Hospital of Oakland Research Institute	Curing Sickle cell Disease with CRISPR-Cas9 genome editing	Therapeutic Translational Research Projects	$4,463,435	
Matthew Porteus	Stanford University	Genome Editing of Autologous Hematopoietic Stem Cells to Treat Sickle Cell Disease	Late Stage Preclinical Projects	$4,849,363	
					Total: $24,757,294.00

CIRM Blood Disease Videos

 **CURED: Stem Cell Clinical Trial Stories**	 **Defeating Sickle Cell Disease with Stem Cells + Gene Therapy**	 **Spotlight on Amyloidosis and Stem Cell Research: Robert Vescio MD - Cedars-Sinai**	 **Michael York: Amyloidosis and Stem Cell Research**
 **William Kim, UCLA - CIRM Stem Cell #SciencePitch**	 **Catriona Jamieson, UCSD - CIRM Stem Cell #SciencePitch**	 **Spotlight on Genomics: Understanding Our Genes**	 **Catriona Jamieson - UCSD | CIRM Spotlight on Genomics**
 **Spotlight on Genomics: Clinical Trial for Myelofibrosis that Targets Cancer Stem Cells**	 **Stem Cell Gene Therapy for Sickle Cell Anemia - Donald Kohn**	 **Spotlight on Basic Research: Irv Weissman**	 **Spotlight on Leukemia: Welcoming Remarks**
 **Spolight on Leukemia: Catriona Jamieson, M.D.**	 **Spotlight on Leukemia: Clinical Trial Participants**	 **Progress and Promise in Leukemia**	

Resources

- NIH: What is Sickle Cell Anemia?
- CDC: Sickle Cell Information
- Find a clinical trial near you: NIH Clinical Trials database

- Sickle Cell Disease Association of America
- Sickle Cell Disease Foundation of California
- American Sickle Cell Anemia Association

Find Out More:
Stem Cell FAQ | Stem Cell Videos | What We Fund

Source URL: https://www.cirm.ca.gov/our-progress/disease-information/sickle-cell-disease-fact-sheet